臨床評価部会総会
Location Flexible Trials
ファイザー R&D 合同会社
ポートフォリオ・プロジェクト・マネジメント統括部
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Our Purpose - Pfizer

Breakthroughs that change patients’ lives
Virtual Clinical Trials (VCTs)

Current Situation
• No official definition of Virtual Clinical Trials (VCTs) yet
• Different types of trials have been started by several companies and academia

Pfizer - Breakthroughs that change patients’ lives

Location Flexible Trials
• Can maximize patient-centricity by allowing the study participant more flexibility in how and where she/he participates in a clinical trial
• Doesn’t have to be an all or nothing approach
Traditional Clinical Trials vs Location Flexible Trials

**Awareness of Clinical Trial**
- Traditional: Introduce/screen by Investigators
- Location Flexible (example): SNS, internet searches by patients

**Informed Consent Process**
- Traditional: Study sites, face-to-face
- Location Flexible (example): Registry software/eConsent

**Dispensing Study Medication**
- Traditional: Study sites
- Location Flexible (example): Direct-to-trial participant shipping

**Data Collection**
- Traditional: Regular site visits/hospital admission
- Location Flexible (example): Collect remotely/local lab, imaging center

**After Clinical Trial**
- Traditional
- Location Flexible (example): Trial experience survey etc.
Why We’re Doing It

• Focuses on the needs of all patients, whoever and wherever they might be
• Improves enrollment and retention ultimately speeding up timelines, so medicine can get to patients faster
• Provides a more diverse and representative patient population

<table>
<thead>
<tr>
<th>80%</th>
<th>1/3</th>
<th>59.8%</th>
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<tbody>
<tr>
<td>of patients would join a trial if the site were within 30 minutes of their home</td>
<td>of all investigative sites enroll zero or one patient in a typical clinical trial</td>
<td>responded the physical location of the study center is important</td>
</tr>
</tbody>
</table>

Key Success Factors

• Engagement with all stakeholders (patients, investigators, PMDA, IRBs, CROs, pharmacies, third-party vendors such as telemedicine or mobile HCPs (e.g., nurses, physicians) during the protocol design process
• Requirements for trial-specific procedures
• Determine who is responsible for the management of source documents at flexible/local sites
• Have clear map of data flow, data storage, and data management plan
• User friendly instruction guide with local language
• Technological support to provide adequate training and troubleshooting for all patients
• Delegation of authority and responsibilities in the context of VCTs (DCTs, LFTs) should not differ from traditional trials
• Keep/ increase clinical trial quality high
First ‘Virtual’ Clinical Trial Allowing Patients to Participate Regardless of Geography in 2011 in the US

REMOTE Trial

Methods: Exploratory, randomized, double-blind, placebo-controlled, parallel-group, single-center, Phase 4 trial to test a novel web-based trial design for evaluating the efficacy and safety of tolterodine ER 4 mg in US participants with OAB

Primary objective: To compare the efficacy of tolterodine ER versus placebo in participants with OAB after 12 weeks of treatment using a web-based trial design

Fig. 2. Electronic data collection and management. EDC = electronic data capture, IVRS = interactive voice response system.

Ref.: M. Orri et al. / Contemporary Clinical Trials 38 (2014) 190-197
Fig. 3. Participant disposition. Percentages at each step of recruitment were calculated using the number of participants who viewed the study introduction web page as the denominator. Percentages at each step of screening were calculated using the number of participants who reconfirmed their e-mail address as the denominator.

Ref.: M. Orri et al. / Contemporary Clinical Trials 38 (2014) 190-197
**Table 1**

Baseline demographic and clinical characteristics.

<table>
<thead>
<tr>
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<th>Placebo (n = 6)</th>
<th>TOL ER (n = 12)</th>
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</thead>
<tbody>
<tr>
<td>Gender, n (%)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>6 (100)</td>
<td>12 (100)</td>
</tr>
<tr>
<td>Mean (range) age, year</td>
<td>46.2 (31–64)</td>
<td>48.4 (28–66)</td>
</tr>
<tr>
<td>Race, n</td>
<td></td>
<td></td>
</tr>
<tr>
<td>White</td>
<td>4</td>
<td>9</td>
</tr>
<tr>
<td>Black</td>
<td>1</td>
<td>2</td>
</tr>
<tr>
<td>Asian</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>OAB/UUI diagnosis, n (%)</td>
<td>6 (100)</td>
<td>12 (100)</td>
</tr>
<tr>
<td>Mean (range) duration</td>
<td>3.5 (1.2–15.7)</td>
<td>3.3 (1.3–30.3)</td>
</tr>
<tr>
<td>since diagnosis, year</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mean (SD) micturitions/24 hours</td>
<td>9.9 (1.7)</td>
<td>11.5 (3.5)</td>
</tr>
</tbody>
</table>

OAB = overactive bladder, SD = standard deviation, TOL ER = tolterodine extended release, UUI = urge urinary incontinence.

**Fig. 4.** Change from baseline to week 12 in micturitions per 24 hours (primary efficacy endpoint). CI = confidence interval, LS = least squares, SE = standard error, TOL ER = tolterodine extended release.
Objectives: “For Better Clinical Trials”

• To clarify Patient Journey (from the first hospital visit to completion of clinical trial or further) of a certain disease, e.g. DMD (Duchenne Muscular Dystrophy), NASH (non-alcoholic steatohepatitis)
• To identify issues and hurdles in terms of patients’ participation in clinical trials
• To propose ideas for providing solutions by leveraging new technology that would improve quality and effectiveness of clinical trials
Voices from the Patients & Their Families

Insights from Workshops with Patients

- Gaining understanding from others (e.g. school teachers, grandparents, and managers at work)
- Informed Consent
- Supporting tools for clinical trials (e.g. concomitant drug information)
- Number of site visits
- Searchability of clinical trial information in Japan
- Thank you letter

Since the full introduction of Virtual Clinical Trials to all clinical trials is not an objective, it is important to think about various approaches that will improve convenience for patients.
Activities Currently We are Doing/ Planning in Japan

Planning

Meet with Regulatory Bodies

Disclose Names of Study Sites

eConsent

Direct-to-Trial Participant Shipping (medication)

Direct-from-Trial Participant Shipping (samples)

After Trial Completion

Patients’ Workshop

Thank You Letter

Patient Lay Summary
Communicate with the patients to get insights about patients’ and their families’ needs, their life, their environment, familiarity with new technology, their preference for how and where they participate in a clinical trial.